



MARCH  
12-14, 2025

Bürgerhaus  
Bahnstadt •  
Gadamer Platz  
1 • 69115  
Heidelberg •  
Germany

Register:



Hosted by:



# CONSENSUS MEETING on Demonstrating Meaningful Change in HSPs and Ataxias & ANNUAL MEETING of TreatHSP and the Tom Wahlig Foundation

Wednesday, March 12, 2025

<b>13:00 – 14:40</b>		
<b>Demonstrating Meaningful Change – State of the Art</b>		
<b>13:00 – 13:10</b>	Welcome and introduction to the programme	Rebecca Schüle, Heidelberg
<b>13:10 – 13:35</b>	Clinical outcome assessments in HSP – current status	Rebecca Schüle
<b>13:35 – 14:00</b>	Clinical outcome assessments in ataxia – current status	Matthis Synofzik, Tübingen
<b>14:00 – 14:40</b>	Preparing the stage: key points from the FDA PFDD guidance on meaningful endpoints	Michele Potashman, Biohaven
<b>14:40 – 15:10</b>	<b>Coffee Break</b>	
<b>15:10 – 17:00</b>		
<b>Demonstrating Meaningful Change – Current Use Cases</b>		
<b>15:10 – 15:35</b>	Anchoring measures for ataxia COAs : experiences with the SARA, PGI, PROM-Ataxia and FARS-ADL	tbc
<b>15:35 – 16:00</b>	Clinical meaningfulness of stride velocity 95th percentile in Duchenne Muscular Dystrophy: pitfalls and learnings from the EMA qualification	tbc
<b>16:00 – 16:30</b>	Establishing meaningful within-patient changes in early Alzheimer’s disease: considerations for anchor selection and anchoring methodologies	tbc
<b>16:30 – 17:00</b>	Application of the PFDD to the validation of the f-SARA and FARS-ADL	Michele Potashman, Biohaven
<b>17:00 – 17:30</b>	<b>Break</b>	



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## Wednesday, March 12, 2025 (ct'd)

### 17:30 – 18:30 Demonstrating Meaningful Change – Round Table Discussion

17:30 – 18:00 Selection of suitable anchor variables for ataxia and HSP All

18:00 – 18:30 Selection of suitable anchoring methods to inform choice of meaningful score difference in ataxia and HSP All

19:30 **Dinner:**  
**Da Baggio, Römerstr. 24-26, 69115 Heidelberg**



## Thursday, March 13, 2025

### 07:00 – 08:00 Registration

### 08:00 – 09:25 Meaningful Change – Specifications and Consensus for HSPs

08:00 – 08:10 Learnings from day 1 Rebecca Schüle, Matthis Synofzik

08:10 – 08:35 Outcome validation study – master protocol for adult patients Rebecca Schüle, all

08:35 – 09:00 Outcome validation study – master protocol for pediatric patients Darius Ebrahimi-Fakhari, Boston, all

09:00 – 09:25 HSP Consensus Paper Series - strategies and responsibilities Rebecca Schüle, all

### 09:25 – 10:40 Meaningful Change – Specifications and Consensus for Ataxias

09:25 – 09:50 Specification and consensus on best ataxia anchors and anchoring methods Matthis Synofzik, all

09:50 – 10:15 Application paths of best anchors & anchoring methods for outcome domains / AGI working groups: ClinRO, DMO, imaging outcomes, molecular outcomes Matthis Synofzik, all

10:15 – 10:40 Writing strategy for AGI consensus paper: establishing meaningful change in ataxia outcomes Matthis Synofzik, all

10:40 – 11:10 **Coffee Break**



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## Thursday, March 13, 2025 (ct'd)

### 11:10 – 12:30 Parallel Session I: News from TreatHSP

11:10 – 11:20	RND Registry – a modular platform for rare disease natural history studies from n-of-1 to n-of-many	Rebecca Schüle
11:20 – 11:30	Mobile gait outcomes in HSP – a window for digital biomarkers	Heiko Gassner, Erlangen
11:30 – 11:40	Investigating axon-specific phenotypes in spastin-deficient iPSC-derived neurons	Stefan Hauser, Tübingen
11:40 – 11:50	Pathophysiology of REEP1 associated axonopathies	Christian Hübner, Jena
11:50 – 12:00	Mutation-specific imaging findings in POLR3A-associated diseases	Inga Harting - tbc
12:00 – 12:10	Human stem cell based disease modeling in HSP and beyond	Beate Winner, Erlangen
12:10 – 12:20	Understanding the function of the ERLIN1/2-RNF170 complex and its role in HSP	Elena Rugarli, Cologne
12:20 – 12:30	Tracking and targeting maladaptive immune reactions in SPG11	Janos Groh, Munich

### 11:10 – 12:30 Parallel Session II: Multicenter ataxia outcome validation studies in selected trial-ready SCAs (end of ataxia-specific programme)

#### 12:30 – 13:30 Lunch Break

### 13:30 – 15:40 Surrogate Endpoints in HSP and Related Diseases

13:30 – 14:00	Surrogate endpoints in Charcot-Marie-Tooth Disease	Mary Reilly, London
14:00 – 14:20	Acetylated alpha-tubulin: a biomarker candidate for SPG4	Gautam Wali, Sydney
14:20 – 14:40	Molecular biomarkers in SPG11: from neurodegeneration to neuroinflammation	Martin Regensburger
14:40 – 15:00	Spinal cord imaging in HSPs: current state and perspectives	Marcondes Franca, Campinas ( <i>remote</i> )
15:00 – 15:20	Brain imaging in HSPs: current state and perspectives	Tobias Lindig, Tübingen
15:20 – 15:40	Peripheral nerve imaging in HSPs: current state and perspectives	Heike Jacobi, Jennifer Hayes, Ann Arbor ( <i>remote</i> )

#### 15:40 – 16:10 Coffee Break



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## Thursday, March 13, 2025 (ct'd)

### 16:10 – 17:10 Poster Flash Talks

16:10 – 16:17	Flash Talk 1	16:10 – 16:17
16:17 – 16:24	Flash Talk 2	16:17 – 16:24
16:24 – 16:31	Flash Talk 3	16:24 – 16:31
16:31 – 16:38	Flash Talk 4	16:31 – 16:38
16:38 – 16:45	Flash Talk 5	16:38 – 16:45
16:45 – 16:52	Flash Talk 6	16:45 – 16:52
16:52 – 16:59	Flash Talk 7	16:52 – 16:59
16:59 – 17:06	Flash Talk 8	16:59 – 17:06

### 17:10 – 18:10 Poster Session

18:20	Leave for the joint walk to the dinner location	Guided by Sabrina Stängle
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**19:00** **Dinner:**  
**Kulturbrauerei Heidelberg,**  
**Leyergasse 6, 69117 Heidelberg**



## Friday, March 14, 2025

### 08:30 – 10:10 Pathways and Therapeutic Targets

08:30 – 08:55	Modulation of PI4K2A activity improves autophagy in SPG11	Christian Hübner
08:55 – 09:20	Emerging molecular-mechanism based therapies for AP-4-associated hereditary spastic paraplegia	Darius Ebrahimi-Fakhari
09:20 – 09:45	Cul-4 inhibition rescues spastin levels and reduces defects in hereditary spastic paraplegia models	Cinzia Rinaldo, Rome
09:45 – 10:10	Small molecule FICD inhibitors suppress endogenous and pathologic FICD-mediated protein AMPylation – implications for HSP	Matthias C. Truttmann, Ann Arbor

**10:10 – 10:40** **Coffee Break**



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## Friday, March 14, 2025 (ct'd)

<b>10:40 – 13:30</b>	<b>Gene and RNA Therapies in HSP</b>	
<b>10:40 – 11:05</b>	Genetics of HSP: genes, mechanisms and challenges	Stephan Züchner, Miami
<b>11:05 – 11:30</b>	Modelling an AAV9 gene therapy for SPG56 ( <i>CYP2U1</i> ) in patient-specific brain organoids	Ernst Wolvetang, Brisbane ( <i>remote</i> )
<b>11:30 – 11:55</b>	Gene replacement therapy for SPG47 ( <i>AP4B1</i> ) – current status of the preclinical development	Mimoun Azzouz, Sheffield
<b>11:55 – 12:20</b>	AAV gene therapy for SPG50 ( <i>AP4M1</i> ): a phase 1 trial in a single patient	tbc
<b>12:20 – 12:45</b>	A gene therapy for SPG50: what it takes	Terry Pirovolakis, Elpida Therapeutics
<b>12:45 – 13:10</b>	Antisense oligonucleotide therapy in an individual with KIF1A-associated neurological disorder	Wendy Chung, Boston
<b>13:10 – 13:30</b>	A drug for Susannah	tbc
<b>13:30 – 14:15</b>	<b>Lunch Break</b>	
<b>14:15 – 16:00</b>	<b>Ethical Challenges in Personalized Genomic Therapies</b>	
<b>14:15 – 14:45</b>	Ethical challenges in personalized genomic therapies	Lynn Bush, Boston ( <i>remote</i> )
<b>14:45 – 15:15</b>	Ethical cost-benefit evaluation and implications for pricing in personalized medicine	tbc
<b>15:15 – 16:00</b>	<b>Award Ceremonies</b>	
<b>15:15 – 15:20</b>	Awarding of the Adolf Strümpell Prize	Gerald Fischer, Euro HSP
<b>15:20 – 15:30</b>	Adolf Strümpell Award Lecture	tbc
<b>15:30 – 15:45</b>	Awarding of the Tom Wahlig Poster Prizes	Henry and Susanne Wahlig
<b>15:45 – 16:00</b>	Farewell and Closing Remarks	Henry and Susanne Wahlig, Rebecca Schüle

End of Meeting